

SEQUENCE LISTING Hildinger, Markus <120> Decreasing gene expression in a mammalian subject in vivo via AAV-mediated RNAi expression cassette transfer <130> 1339 <140> US 10/604,340 <141> 2003-07-13 <160> 11 <170> PatentIn version 3.1 <210> 1 <211> 6437 <212> DNA <213> Artificial <220> <223> sequence for recombinant adeno-associated viral vector, including plasmid backbone, with AAV2 internal terminal repeats that flank expression cassette; referred to as AAV2/2 CMV luciferase <220> <221> CDS <222> (1228)..(2883)<223> luciferase cDNA <400> 1 agegeceaat aegeaaaceg ceteteceeg egegttggec gatteattaa tgeagetgge 60 acgacaggtt tcccgactgg aaagcgggca gtgagcgcaa cgcaattaat gtgagttagc 120 tcactcatta ggcaccccag gctttacact ttatgcttcc ggctcgtatg ttgtgtggaa 180 ttgtgagcgg ataacaattt cacacaggaa acagctatga ccatgattac gccagattta 240 attaaggctg cgcgctcgct cgctcactga ggccgcccgg gcaaagcccg ggcgtcgggc 300 360 gacetttggt egeeeggeet eagtgagega gegagegeg agagagggag tggeeaacte catcactagg ggttccttgt agttaatgat taacccgcca tgctacttat ctacgtagcc 420 atgctctagg aagatcggaa ttcgccctta agctagctag ttattaatag taatcaatta 480 cggggtcatt agttcatagc ccatatatgg agttccgcgt tacataactt acggtaaatg 540 qcccqcctqq ctqaccqccc aacqaccccc qcccattgac qtcaataatq acqtatqttc 600

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- <212> PRT
- <213> Artificial

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<223> sequence for recombinant adeno-associated viral vector, including plasmid backbone, with AAV2 internal terminal repeats that flank expression cassette; referred to as AAV2/2 CMV luciferase

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Val Asp Ile Thr Tyr Ala Glu Tyr Phe Glu Met Ser Val Arg Leu Ala 50 60

Glu Ala Met Lys Arg Tyr Gly Leu Asn Thr Asn His Arg Ile Val Val 65 70 75 80

Cys Ser Glu Asn Ser Leu Gln Phe Phe Met Pro Val Leu Gly Ala Leu 85 90 95

Phe Ile Gly Val Ala Val Ala Pro Ala Asn Asp Ile Tyr Asn Glu Arg 100 105 110

Glu Leu Leu Asn Ser Met Gly Ile Ser Gln Pro Thr Val Val Phe Val
115 120 125

Ser Lys Lys Gly Leu Gln Lys Ile Leu Asn Val Gln Lys Lys Leu Pro 130 140

Ile Ile Gln Lys Ile Ile Met Asp Ser Lys Thr Asp Tyr Gln Gly
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Phe Gln Ser Met Tyr Thr Phe Val Thr Ser His Leu Pro Pro Gly Phe 165 170 175

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Pro	Glu	Gly 355	Asp	Asp	Lys	Pro	Gly 360	Ala	Val	Gly	Lys	Val 365	Val	Pro	Phe
Phe	Glu 370	Ala	Lys	Val	Val	Asp 375	Leu	Asp	Thr	Gly	Lys 380	Thr	Leu	Gly	Val
Asn 385	Gln	Arg	Gly	Glu	Leu 390	Cys	Val	Arg	Gly	Pro 395	Met	Ile	Met	Ser	Gly 400

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Trp Leu His Ser Gly Asp Ile Ala Tyr Trp Asp Glu Asp Glu His Phe 420 425 430

Phe Ile Val Asp Arg Leu Lys Ser Leu Ile Lys Tyr Lys Gly Tyr Gln 435 440 445

Val Ala Pro Ala Glu Leu Glu Ser Ile Leu Leu Gln His Pro Asn Ile 450 455 460

Phe Asp Ala Gly Val Ala Gly Leu Pro Asp Asp Asp Ala Gly Glu Leu 465 470 475 480

Pro Ala Ala Val Val Leu Glu His Gly Lys Thr Met Thr Glu Lys
485 490 495

Glu Ile Val Asp Tyr Val Ala Ser Gln Val Thr Thr Ala Lys Leu
500 505 510

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Gly Gly Lys Ile Ala Val 545 550

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<223> sequence for recombinant adeno-associated viral vector, including
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 expression cassette; referred to as AAV2/5 CMV luciferase

<220>

<221> CDS

<222> (1228)..(2883)

<223> luciferase cDNA

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<210> 4

<211> 550

<212> PRT

<213> Artificial

<220>

<223> sequence for recombinant adeno-associated viral vector, including
 plasmid backbone, with AAV2 internal terminal repeats that flank
 expression cassette; referred to as AAV2/5 CMV luciferase

<400> 4

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Leu Glu Asp Gly Thr Ala Gly Glu Gln Leu His Lys Ala Met Lys Arg 20 25 30

Tyr Ala Leu Val Pro Gly Thr Ile Ala Phe Thr Asp Ala His Ile Glu 35 40 45

Val Asp Ile Thr Tyr Ala Glu Tyr Phe Glu Met Ser Val Arg Leu Ala 50 55 60

Glu Ala Met Lys Arg Tyr Gly Leu Asn Thr Asn His Arg Ile Val Val 65 70 75 80

Cys Ser Glu Asn Ser Leu Gln Phe Phe Met Pro Val Leu Gly Ala Leu 85 90 95

Phe Ile Gly Val Ala Val Ala Pro Ala Asn Asp Ile Tyr Asn Glu Arg 100 105 110

Glu Leu Leu Asn Ser Met Gly Ile Ser Gln Pro Thr Val Val Phe Val Ser Lys Lys Gly Leu Gln Lys Ile Leu Asn Val Gln Lys Lys Leu Pro Ile Ile Gln Lys Ile Ile Ile Met Asp Ser Lys Thr Asp Tyr Gln Gly Phe Gln Ser Met Tyr Thr Phe Val Thr Ser His Leu Pro Pro Gly Phe Asn Glu Tyr Asp Phe Val Pro Glu Ser Phe Asp Arg Asp Lys Thr Ile Ala Leu Ile Met Asn Ser Ser Gly Ser Thr Gly Leu Pro Lys Gly Val Ala Leu Pro His Arg Thr Ala Cys Val Arg Phe Ser His Ala Arg Asp Pro Ile Phe Gly Asn Gln Ile Ile Pro Asp Thr Ala Ile Leu Ser Val Val Pro Phe His His Gly Phe Gly Met Phe Thr Thr Leu Gly Tyr Leu Ile Cys Gly Phe Arg Val Val Leu Met Tyr Arg Phe Glu Glu Leu Phe Leu Arg Ser Leu Gln Asp Tyr Lys Ile Gln Ser Ala Leu Leu Val Pro Thr Leu Phe Ser Phe Phe Ala Lys Ser Thr Leu Ile Asp Lys Tyr Asp Leu Ser Asn Leu His Glu Ile Ala Ser Gly Gly Ala Pro Leu Ser Lys Glu Val Gly Glu Ala Val Ala Lys Arg Phe His Leu Pro Gly Ile

Arg Gln Gly Tyr Gly Leu Thr Glu Thr Thr Ser Ala Ile Leu Ile Thr Pro Glu Gly Asp Asp Lys Pro Gly Ala Val Gly Lys Val Val Pro Phe Phe Glu Ala Lys Val Val Asp Leu Asp Thr Gly Lys Thr Leu Gly Val Asn Gln Arg Gly Glu Leu Cys Val Arg Gly Pro Met Ile Met Ser Gly Tyr Val Asn Asn Pro Glu Ala Thr Asn Ala Leu Ile Asp Lys Asp Gly Trp Leu His Ser Gly Asp Ile Ala Tyr Trp Asp Glu Asp Glu His Phe Phe Ile Val Asp Arg Leu Lys Ser Leu Ile Lys Tyr Lys Gly Tyr Gln Val Ala Pro Ala Glu Leu Glu Ser Ile Leu Leu Gln His Pro Asn Ile Phe Asp Ala Gly Val Ala Gly Leu Pro Asp Asp Ala Gly Glu Leu Pro Ala Ala Val Val Leu Glu His Gly Lys Thr Met Thr Glu Lys Glu Ile Val Asp Tyr Val Ala Ser Gln Val Thr Thr Ala Lys Lys Leu Arg Gly Gly Val Val Phe Val Asp Glu Val Pro Lys Gly Leu Thr Gly Lys Leu Asp Ala Arg Lys Ile Arg Glu Ile Leu Ile Lys Ala Lys Lys Gly Gly Lys Ile Ala Val

- <210> 5
- <211> 3618
- <212> DNA
- <213> Artificial

<220>

<223> sequence for recombinant adeno-associated viral vector, including
 plasmid backbone, with AAV2 internal terminal repeats that flank
 expression cassette; referred to as AAV2/5 U6 lucRI-la

<400> 5 agogoccaat acgcaaaccg cototocccq cgcqttqqcc qattcattaa tqcaqctqqc 60 acgacaggtt tcccgactgg aaagcgggca gtgagcgcaa cgcaattaat gtgagttagc 120 tcactcatta ggcaccccag gctttacact ttatgcttcc ggctcgtatg ttgtgtggaa 180 ttgtgagcgg ataacaattt cacacaggaa acagctatga ccatgattac gccagattta 240 attaaggetg egegeteget egeteactga ggeegeeegg geaaageeeg ggegteggge 300 gacetttggt egeeeggeet eagtgagega gegagegege agagagggag tggeeaacte 360 catcactagg ggttccttgt agttaatgat taacccgcca tgctacttat ctacgtagcc 420 atgetetagg aagateggaa ttegeeetta agetageeee cagtggaaag aegegeagge 480 aaaacgcacc acgtgacgga gcgtgaccgc gcgccgagcc caaggtcggg caggaagagg 540 gcctatttcc catgattcct tcatatttgc atatacgata caaggctgtt agagagataa 600 ttagaattaa tttgactgta aacacaaaga tattagtaca aaatacgtga cgtagaaagt 660 aataatttct tgggtagttt gcagttttaa aattatgttt taaaatggac tatcatatgc 720 ttaccgtaac ttgaaagtat ttcgatttct tggctttata tatcttgtgg aaaggacgaa 780 acaccettae getgagtaet tegatteaag agategaagt aeteagegta agtttttete 840 gagttaaggg cgaattcccg attaggatct tcctagagca tggctacgta gataagtagc 900 atggcgggtt aatcattaac tacaaggaac ccctagtgat ggagttggcc actccctctc 960 tgcgcgctcg ctcgctcact gaggccgggc gaccaaaggt cgcccgacgc ccgggctttg 1020 eccgggegge etcagtgage gagegagege geageettaa ttaacetaat teaetggeeg 1080 tegttttaca aegtegtgae tgggaaaaee etggegttae ecaaettaat egeettgeag 1140 1200 cacatecece tttegecage tggegtaata gegaagagge cegeacegat egecetteee aacaqttqcq caqcctqaat qqcqaatqqq acqcqcctq taqcqqcqca ttaaqcqcqq 1260 egggtgtggt ggttaegege agegtgaeeg etaeaettge eagegeeeta gegeeegete 1320 ctttcgcttt cttcccttcc tttctcgcca cgttcgccgg ctttccccgt caagctctaa 1380 atcgggggct ccctttaggg ttccgattta gtgctttacg gcacctcgac cccaaaaaac 1440 ttgattaggg tgatggttca cgtagtgggc catcgccccg atagacggtt tttcgccctt 1500 tgacgctgga gttcacgttc ctcaatagtg gactcttgtt ccaaactgga acaacactca 1560 1620 accetatete ggtetattet tttgatttat aagggatttt teegattteg geetattggt 1680 taaaaaatga gctgatttaa caaaaattta acgcgaattt taacaaaata ttaacgttta 1740 taatttcagg tggcatcttt cggggaaatg tgcgcggaac ccctatttgt ttattttct aaatacatto aaatatgtat cogotoatga gacaataaco otgataaatg ottoaataat 1800 1860 attgaaaaag gaagagtatg agtattcaac atttccgtgt cgcccttatt cccttttttg eggeattttg cetteetgtt tttgeteace cagaaacget ggtgaaagta aaagatgetg 1920 aagatcagtt gggtgcacga gtgggttaca tcgaactgga tctcaatagt ggtaagatcc 1980 2040 ttgagagttt tcgccccgaa gaacgttttc caatgatgag cacttttaaa gttctgctat 2100 gtggcgcggt attatcccgt attgacgccg ggcaagagca actcggtcgc cgcatacact 2160 attotoagaa tgacttggtt gagtactoac cagtoacaga aaagcatott acggatggca 2220 tgacagtaag agaattatgc agtgctgcca taaccatgag tgataacact gcggccaact 2280 tacttctgac aacgatcgga ggaccgaagg agctaaccgc ttttttgcac aacatggggg atcatgtaac tegeettgat egttgggaac eggagetgaa tgaageeata eeaaaegaeg 2340 2400 agegtgacae caegatgeet gtagtaatgg taacaaegtt gegcaaacta ttaactggeg aactacttac tctagcttcc cggcaacaat taatagactg gatggaggcg gataaagttg 2460 caggaccact tctgcgctcg gcccttccgg ctggctggtt tattgctgat aaatctggag 2520 ccggtgagcg tgggtctcgc ggtatcattg cagcactggg gccagatggt aagccctccc 2580 2640 gtatcgtagt tatctacacg acggggagtc aggcaactat ggatgaacga aatagacaga 2700 tegetgagat aggtgeetea etgattaage attggtaact gteagaceaa gtttaeteat 2760 tttttgataa tctcatgacc aaaatccctt aacgtgagtt ttcgttccac tgagcgtcag 2820 accccgtaga aaagatcaaa ggatcttctt gagatccttt ttttctgcgc gtaatctgct 2880 2940 gcttgcaaac aaaaaaacca ccgctaccag cggtggtttg tttgccggat caagagctac caactetttt teegaaggta aetggettea geagagegea gataceaaat aetgteette 3000 tagtgtagcc gtagttaggc caccacttca agaactctgt agcaccgcct acatacctcg 3060 3120 ctctgctaat cctgttacca gtggctgctg ccagtggcga taagtcgtgt cttaccgggt

3180 tggactcaag acgatagtta ccggataagg cgcagcggtc gggctgaacg gggggttcgt gcacacagcc cagcttggag cgaacgacct acaccgaact gagataccta cagcgtgagc 3240 tatgagaaag cgccacgctt cccgaaggga gaaaggcgga caggtatccg gtaagcggca 3300 3360 gggtcggaac aggagagcgc acgagggagc ttccaggggg aaacgcctgg tatctttata gtcctgtcgg gtttcgccac ctctgacttg agcgtcgatt tttgtgatgc tcgtcagggg 3420 ggcggagcct atggaaaaac gccagcaacg cggccttttt acggttcctg gccttttgct 3480 gcggttttgc tcacatgttc tttcctgcgt tatcccctga ttctgtggat aaccgtatta 3540 ccgcctttga gtgagctgat accgctcgcc gcagccgaac gaccgagcgc agcgagtcag 3600 tgagcgagga agcggaag 3618

<210> 6

<211> 3920

<212> DNA

<213> Artificial

<220>

<223> sequence for recombinant adeno-associated viral vector, including
 plasmid backbone, with AAV2 internal terminal repeats that flank
 expression cassette; referred to as AAV2/5 U6 lucRI-1b

<400> 6 agggcccaat acgcaaaccg cctctccccq cgcgttqgcc gattcattaa tqcaqctqqc 60 acqacagqtt tcccqactqq aaaqcqqqca qtqaqcqcaa cqcaattaat qtqaqttaqc 120 tcactcatta ggcaccccag gctttacact ttatgcttcc ggctcgtatg ttgtgtggaa 180 ttgtgagcgg ataacaattt cacacaggaa acagctatga ccatgattac gccagattta 240 attaaggetg egegeteget egeteactga ggeegeeegg geaaageeeg ggegteggge 300 360 gacetttggt egeeeggeet eagtgagega gegagegege agagagggag tggeeaacte catcactagg ggttccttgt agttaatgat taacccgcca tgctacttat ctacgtagcc 420 atgctctagg aagatcggaa ttcgccctta agctagctag ttattaatag taatcaatta 480 cggggtcatt agttcatagc ccatatatgg agttccgcgt tacataactt acggtaaatg 540 gcccgcctgg ctgaccgccc aacgaccccc gcccattgac gtcaataatg acgtatgttc 600 ccatagtaac gccaataggg actttccatt gacgtcaatg ggtggagtat ttacggtaaa 660 ctgcccactt ggcagtacat caagtgtatc atatgccaag tacgccccct attgacgtca 720 atgacggtaa atggcccgcc tggcattatg cccagtacat gaccttatgg gactttccta 780

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<210> 7

<211> 3923

<212> DNA

<213> Artificial

<220>

<223> sequence for recombinant adeno-associated viral vector, including

plasmid backbone, with AAV2 internal terminal repeats that flank expression cassette; referred to as AAV2/5 U6/U6 lucRIU6-3

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tcactcatta	ggcaccccag	gctttacact	ttatgcttcc	ggctcgtatg	ttgtgtggaa	180
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cgctcctttc gctttcttcc cttcctttct cgccacgttc gccggctttc cccgtcaagc 1680 tctaaatcgg gggctccctt tagggttccg atttagtgct ttacggcacc tcgaccccaa 1740 aaaacttgat tagggtgatg gttcacgtag tgggccatcg ccccgataga cggtttttcg 1800 1860 ccctttgacg ctggagttca cgttcctcaa tagtggactc ttgttccaaa ctggaacaac 1920 actcaaccct atctcggtct attcttttga tttataaggg atttttccga tttcggccta 1980 ttggttaaaa aatgagctga tttaacaaaa atttaacgcg aattttaaca aaatattaac 2040 gtttataatt tcaggtggca tctttcgggg aaatgtgcgc ggaaccccta tttgtttatt tttctaaata cattcaaata tgtatccgct catgagacaa taaccctgat aaatgcttca 2100 ataatattga aaaaggaaga gtatgagtat tcaacatttc cgtgtcgccc ttattccctt 2160 ttttgcggca ttttgccttc ctgtttttgc tcacccagaa acgctggtga aagtaaaaga 2220 2280 tgctgaagat cagttgggtg cacgagtggg ttacatcgaa ctggatctca atagtggtaa gatecttgag agttttcgcc ccgaagaacg ttttccaatg atgagcactt ttaaagttct 2340 2400 gctatgtggc gcggtattat cccgtattga cgccgggcaa gagcaactcg gtcgccgcat acactattct cagaatgact tggttgagta ctcaccagtc acagaaaagc atcttacgga 2460 2520 tggcatgaca gtaagagaat tatgcagtgc tgccataacc atgagtgata acactgcggc 2580 caacttactt ctgacaacga tcggaggacc gaaggagcta accgcttttt tgcacaacat 2640 gggggatcat gtaactcgcc ttgatcgttg ggaaccggag ctgaatgaag ccataccaaa 2700 cgacgagcgt gacaccacga tgcctgtagt aatggtaaca acgttgcgca aactattaac 2760 tggcgaacta cttactctag cttcccggca acaattaata gactggatgg aggcggataa 2820 agttgcagga ccacttctgc gctcggccct tccggctggc tggtttattg ctgataaatc 2880 tggagccggt gagcgtgggt ctcgcggtat cattgcagca ctggggccag atggtaagcc ctcccgtatc gtagttatct acacgacggg gagtcaggca actatggatg aacgaaatag 2940 3000 acagateget gagataggtg ceteactgat taageattgg taactgteag accaagttta ctcatatata ctttagattg atttaaaact tcatttttaa tttaaaagga tctaggtgaa 3060 3120 gatccttttt gataatctca tgaccaaaat cccttaacgt gagttttcgt tccactgagc gtcagacccc gtagaaaaga tcaaaggatc ttcttgagat ccttttttc tgcgcgtaat 3180 3240 ctgctgcttg caaacaaaaa aaccaccgct accagcggtg gtttgtttgc cggatcaaga 3300 gctaccaact ctttttccga aggtaactgg cttcagcaga gcgcagatac caaatactgt

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<210> 8

<211> 3589

<212> DNA

<213> Artificial

<220>

<223> sequence for recombinant adeno-associated viral vector, including
 plasmid backbone, with AAV2 internal terminal repeats that flank
 expression cassette; referred to as AAV2/5 U6 lucRI-4(sense)

<400> 8 agegeecaat aegeaaaceg ceteteeceg egegttggee gatteattaa tgeagetgge 60 acgacaggtt tecegactgg aaagegggea gtgagegeaa egeaattaat gtgagttage 120 teacteatta ggeaceceag getttaeact ttatgettee ggetegtatg ttgtgtggaa 180 ttgtgagcgg ataacaattt cacacaggaa acagctatga ccatgattac gccagattta 240 300 attaaggetg egegeteget egeteactga ggeegeeegg geaaageeeg ggegteggge gacctttggt cgcccggcct cagtgagcga gcgagcgcgc agagagggag tggccaactc 360 catcactagg ggttccttgt agttaatgat taacccgcca tgctacttat ctacqtagcc 420 atgetetagg aagateggaa ttegeeetta agetageeee cagtggaaag aegegeagge 480 540 aaaacgcacc acgtgacgga gcgtgaccgc gcgccgagcc caaggtcggg caggaagagg gcctatttcc catgattcct tcatatttgc atatacgata caaggctgtt agagagataa 600 660 ttagaattaa tttgactgta aacacaaaga tattagtaca aaatacgtga cgtagaaagt 720 aataatttct tgggtagttt gcagttttaa aattatgttt taaaatggac tatcatatgc

780 ttaccgtaac ttgaaagtat ttcgatttct tggctttata tatcttgtgg aaaggacgaa 840 acaccettae getgagtaet tegattttet egagttaagg gegaatteee gattaggate 900 ttcctagagc atggctacgt agataagtag catggcgggt taatcattaa ctacaaggaa 960 cccctagtga tggagttggc cactccctct ctgcgcgctc gctcgctcac tgaggccggg 1020 cgaccaaagg tcgcccgacg cccgggcttt gcccgggcgg cctcagtgag cgagcgagcg 1080 cgcagcctta attaacctaa ttcactggcc gtcgttttac aacgtcgtga ctgggaaaac cctggcgtta cccaacttaa tcgccttgca gcacatcccc ctttcgccag ctggcgtaat 1140 agcgaagagg cccgcaccga tcgcccttcc caacagttgc gcagcctgaa tggcgaatgg 1200 1260 gacgcgccct gtagcggcgc attaagcgcg gcgggtgtgg tggttacgcg cagcgtgacc gctacacttg ccagcgccct agcgcccgct cctttcgctt tcttcccttc ctttctcgcc 1320 1380 acgttcgccg gctttccccg tcaagctcta aatcgggggc tccctttagg gttccgattt 1440 agtgctttac ggcacctcga ccccaaaaaa cttgattagg gtgatggttc acgtagtggg 1500 ccatcgcccc gatagacggt ttttcgccct ttgacgctgg agttcacgtt cctcaatagt 1560 ggactettgt tecaaactgg aacaacacte aaccetatet eggtetatte tittgattta taagggattt ttccgatttc ggcctattgg ttaaaaaatg agctgattta acaaaaattt 1620 1680 aacgcgaatt ttaacaaaat attaacgttt ataatttcag gtggcatctt tcggggaaat gtgcgcggaa cccctatttg tttatttttc taaatacatt caaatatgta tccgctcatg 1740 1800 agacaataac cctgataaat gcttcaataa tattgaaaaa ggaagagtat gagtattcaa 1860 cattleegtg tegecettat teeetttttt geggeatttt geetteetgt ttttgeteae 1920 ccagaaacgc tggtgaaagt aaaagatgct gaagatcagt tgggtgcacg agtgggttac 1980 atcgaactgg atctcaatag tggtaagatc cttgagagtt ttcgccccga agaacgtttt 2040 ccaatgatga gcacttttaa agttctgcta tgtggcgcgg tattatcccg tattgacgcc 2100 gggcaagagc aactcggtcg ccgcatacac tattctcaga atgacttggt tgagtactca 2160 ccagtcacag aaaagcatct tacggatggc atgacagtaa gagaattatg cagtgctgcc 2220 ataaccatga gtgataacac tgcggccaac ttacttctga caacgatcgg aggaccgaag gagctaaccg cttttttgca caacatgggg gatcatgtaa ctcgccttga tcgttgggaa 2280 ccggagctga atgaagccat accaaacgac gagcgtgaca ccacgatgcc tgtagtaatg 2340 gtaacaacgt tgcgcaaact attaactggc gaactactta ctctagcttc ccggcaacaa 2400

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ttatcccctg	attctgtgga	taaccgtatt	accgcctttg	agtgagctga	taccgctcgc	3540
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<210> 9

<211> 3589

<212> DNA

<213> Artificial

<220>

<223> sequence for recombinant adeno-associated viral vector, including
 plasmid backbone, with AAV2 internal terminal repeats that flank
 expression cassette; referred to as AAV2/5 U6 lucRI-4(antisense)

<400> 9
agcgcccaat acgcaaaccg cctctccccg cgcgttggcc gattcattaa tgcagctggc 60
acgacaggtt tcccgactgg aaagcgggca gtgagcgcaa cgcaattaat gtgagttagc 120
tcactcatta ggcaccccag gctttacact ttatgcttcc ggctcgtatg ttgtgtggaa 180

ttgtgagcgg ataacaattt cacacaggaa acagctatga ccatgattac gccagattta 240 attaaggctg cgcgctcgct cgctcactga ggccgcccgg gcaaagcccg ggcgtcgggc 300 360 gacctttggt cgcccggcct cagtgagcga gcgagcgcgc agagagggag tggccaactc catcactagg ggttccttgt agttaatgat taacccgcca tgctacttat ctacgtagcc 420 atgctctagg aagatcggaa ttcgccctta agctagcccc cagtggaaag acgcgcaggc 480 aaaacgcacc acgtgacgga gcgtgaccgc gcgccgagcc caaggtcggg caggaagagg 540 gcctatttcc catgattcct tcatatttgc atatacgata caaggctgtt agagagataa 600 ttagaattaa tttgactgta aacacaaaga tattagtaca aaatacgtga cgtagaaagt 660 720 aataatttct tgggtagttt gcagttttaa aattatgttt taaaatggac tatcatatgc ttaccgtaac ttgaaagtat ttcgatttct tggctttata tatcttgtgg aaaggacgaa 780 acacctcgaa gtactcagcg taagttttct cgagttaagg gcgaattccc gattaggatc 840 900 ttcctagagc atggctacgt agataagtag catggcgggt taatcattaa ctacaaggaa cccctagtga tggagttggc cactccctct ctgcgcgctc gctcgctcac tgaggccggg 960 1020 cgaccaaagg tegecegacg ecegggettt geeegggegg eeteagtgag egagegageg cgcagcctta attaacctaa ttcactggcc gtcgttttac aacgtcgtga ctgggaaaac 1080 1140 cctggcgtta cccaacttaa tcgccttgca gcacatcccc ctttcgccag ctggcgtaat agegaagagg ceegeacega tegecettee caacagttge geageetgaa tggegaatgg 1200 gacgcgccct gtagcggcgc attaagcgcg gcgggtgtgg tggttacgcg cagcgtgacc 1260 gctacacttg ccagcgccct agcgcccgct cctttcgctt tcttcccttc ctttctcgcc 1320 1380 acgttcgccg gctttccccg tcaagctcta aatcgggggc tccctttagg gttccgattt 1440 agtgctttac ggcacctcga ccccaaaaaa cttgattagg gtgatggttc acgtagtggg 1500 ccatcgcccc gatagacggt ttttcgccct ttgacgctgg agttcacgtt cctcaatagt 1560 ggactettgt tecaaactgg aacaacacte aaccetatet eggtetatte ttttgattta 1620 taagggattt ttccgatttc ggcctattgg ttaaaaaatg agctgattta acaaaaattt 1680 aacgcgaatt ttaacaaaat attaacgttt ataatttcag gtggcatctt tcggggaaat 1740 gtgcgcggaa cccctatttg tttatttttc taaatacatt caaatatgta tccgctcatg 1800 agacaataac cctgataaat gcttcaataa tattgaaaaa ggaagagtat gagtattcaa catttccgtg tcgcccttat tccctttttt gcggcatttt gccttcctgt ttttgctcac 1860

1920 ccagaaacgc tggtgaaagt aaaagatgct gaagatcagt tgggtgcacg agtgggttac 1980 atcgaactgg atctcaatag tggtaagatc cttgagagtt ttcgccccga agaacgtttt 2040 ccaatgatga gcacttttaa agttctgcta tgtggcgcgg tattatcccg tattgacgcc gggcaagage aacteggteg cegeatacae tatteteaga atgaettggt tgagtaetea 2100 ccagtcacag aaaagcatct tacggatggc atgacagtaa gagaattatg cagtgctgcc 2160 ataaccatga gtgataacac tgcggccaac ttacttctga caacgatcgg aggaccgaag 2220 gagctaaccg cttttttgca caacatgggg gatcatgtaa ctcgccttga tcgttgggaa 2280 ccggagctga atgaagccat accaaacgac gagcgtgaca ccacgatgcc tgtagtaatg 2340 gtaacaacgt tgcgcaaact attaactggc gaactactta ctctagcttc ccggcaacaa 2400 ttaatagact ggatggaggc ggataaagtt gcaggaccac ttctgcgctc ggcccttccg 2460 gctggctggt ttattgctga taaatctgga gccggtgagc gtgggtctcg cggtatcatt 2520 gcagcactgg ggccagatgg taagccctcc cgtatcgtag ttatctacac gacggggagt 2580 caggcaacta tggatgaacg aaatagacag atcgctgaga taggtgcctc actgattaag 2640 2700 cattggtaac tgtcagacca agtttactca tatatacttt agattgattt aaaacttcat ttttaattta aaaggatcta ggtgaagatc ctttttgata atctcatgac caaaatccct 2760 taacgtgagt tttcgttcca ctgagcgtca gaccccgtag aaaagatcaa aggatcttct 2820 tgagateett tttttetgeg egtaatetge tgettgeaaa caaaaaaace acegetacea 2880 gcggtggttt gtttgccgga tcaagagcta ccaactcttt ttccgaaggt aactggcttc 2940 agcagagcgc agataccaaa tactgtcctt ctagtgtagc cgtagttagg ccaccacttc 3000 aagaactetg tagcacegee tacatacete getetgetaa teetgttaee agtggetget 3060 gccagtggcg ataagtcgtg tcttaccggg ttggactcaa gacgatagtt accggataag 3120 gcgcagcggt cgggctgaac ggggggttcg tgcacacagc ccagcttgga gcgaacgacc 3180 tacaccgaac tgagatacct acagcgtgag ctatgagaaa gcgccacgct tcccgaaggg 3240 3300 agaaaggcgg acaggtatcc ggtaagcggc agggtcggaa caggagagcg cacgagggag cttccagggg gaaacgcctg gtatctttat agtcctgtcg ggtttcgcca cctctgactt 3360 3420 gagcgtcgat ttttgtgatg ctcgtcaggg gggcggagcc tatggaaaaa cgccagcaac geggeetttt taeggtteet ggeettttge tgeggttttg etcacatgtt ettteetgeg 3480 ttatcccctg attctgtgga taaccgtatt accgcctttg agtgagctga taccgctcgc 3540 cgcagccgaa cgaccgagcg cagcgagtca gtgagcgagg aagcggaag 3589

- <210> 10
- <211> 3617
- <212> DNA
- <213> Artificial

<220>

<223> sequence for recombinant adeno-associated viral vector, including plasmid backbone, with AAV2 internal terminal repeats that flank expression cassette; referred to as AAV2/2 U6 eGFPRI-1a

<400> 10 agogoccaat acgcaaaccg cototocccg cgcgttggcc gattcattaa tgcagctggc 60 acgacaggtt tcccgactgg aaagcgggca gtgagcgcaa cgcaattaat gtgagttagc 120 tcactcatta ggcaccccag gctttacact ttatgcttcc ggctcgtatg ttgtgtggaa 180 ttgtgagcgg ataacaattt cacacaggaa acagctatga ccatgattac gccagattta 240 attaaggetg egegeteget egeteactga ggeegeeegg geaaageeeg ggegteggge 300 gacctttggt cgcccggcct cagtgagcga gcgagcgcgc agagagggag tggccaactc 360 catcactagg ggttccttgt agttaatgat taacccgcca tgctacttat ctacgtagcc 420 atgetetagg aagateggaa ttegeeetta agetageeee cagtggaaag aegegeagge 480 aaaacqcacc acqtqacqqa qcqtqaccqc qcqccqaqcc caaqqtcqqq caqqaaqaqq 540 600 gcctatttcc catgattcct tcatatttgc atatacgata caaggctgtt agagagataa ttagaattaa tttgactgta aacacaaaga tattagtaca aaatacgtga cgtagaaagt 660 aataatttct tgggtagttt gcagttttaa aattatgttt taaaatggac tatcatatgc 720 ttaccgtaac ttgaaagtat ttcgatttct tggctttata tatcttgtgg aaaggacgaa 780 acaccgaaga agtcgtgctg cttcttcaag agagaagcag cacgacttct tcttttctcg 840 900 agttaagggc gaattcccga ttaggatctt cctagagcat ggctacgtag ataagtagca tggcgggtta atcattaact acaaggaacc cctagtgatg gagttggcca ctccctctct 960 gegegetege tegeteactg aggeegggeg accaaaggte geeegaegee egggetttge 1020 ccgggcggcc tcagtgagcg agcgagcgcg cagccttaat taacctaatt cactggccgt 1080 cgttttacaa cgtcgtgact gggaaaaccc tggcgttacc caacttaatc gccttgcagc 1140 acateceect ttegecaget ggegtaatag egaagaggee egeacegate geeetteeca 1200 acagttgcgc agcctgaatg gcgaatggga cgcgcctgt agcggcgcat taagcgcggc 1260 gggtgtggtg gttacqcgca gcgtgaccgc tacacttgcc agcgccctag cqcccqctcc 1320

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cggttttgct	cacatgttct	ttcctgcgtt	atcccctgat	tctgtggata	accgtattac	3540
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gagcgaggaa	gcggaag					3617

<210> 11

<211> 3787

<212> DNA

<213> Artificial

<220>

<223> sequence for recombinant adeno-associated viral vector, including
 plasmid backbone, with AAV2 internal terminal repeats that flank
 expression cassette; referred to as AAV2/5 poll lucRI

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